Ruxolitinib in corticosteroid-refractory
graft-versus-host disease after
allogeneic stem cell transplantation: a
multicenter survey.

Although major improvements in
allogeneic hematopoietic cell
transplantation over the past decades,
corticosteroid-refractory (SR) acute (a) and chronic (c) graft-versus-host
disease (GVHD) cause high mortality. Preclinical evidence indicates the
potent anti-inflammatory properties of the JAK1/2 inhibitor ruxolitinib. In this
retrospective survey, 19 stem cell
transplant centers in Europe and the
United States reported outcome data
from 95 patients who had received
ruxolitinib as salvage therapy for
SR-GVHD. Patients were classified as
having SR-aGVHD (n=54, all grades
III or IV) or SR-cGVHD (n=41, all
moderate or severe). The median
number of previous GVHD-therapies
was 3 for both SR-aGVHD (1-7) and
SR-cGVHD (1-10). The overall
response rate was 81.5% (44/54) in
SR-aGVHD including 25 complete
responses (46.3%), while for
SR-cGVHD the ORR was 85.4% (35/41). Of those patients responding to ruxolitinib, the rate of GVHD-relapse was 6.8% (3/44) and 5.7% (2/35) for SR-aGVHD and SR-cGVHD, respectively. The 6-month-survival was 79% (67.3-90.7%, 95% confidence interval (CI)) and 97.4% (92.3-100%, 95% CI) for SR-aGVHD and SR-cGVHD, respectively. Cytopenia and cytomegalovirus-reactivation were observed during ruxolitinib treatment in both SR-aGVHD (30/54, 55.6% and 18/54, 33.3%) and SR-cGVHD (7/41, 17.1% and 6/41, 14.6%) patients. Ruxolitinib may constitute a promising new treatment option for SR-aGVHD and SR-cGVHD that should be validated in a prospective trial.